

Fig. 1a

Non-Transduced hMSCs in Immuno-competent

mouse muscle

Day 3

DiI + hMSCs

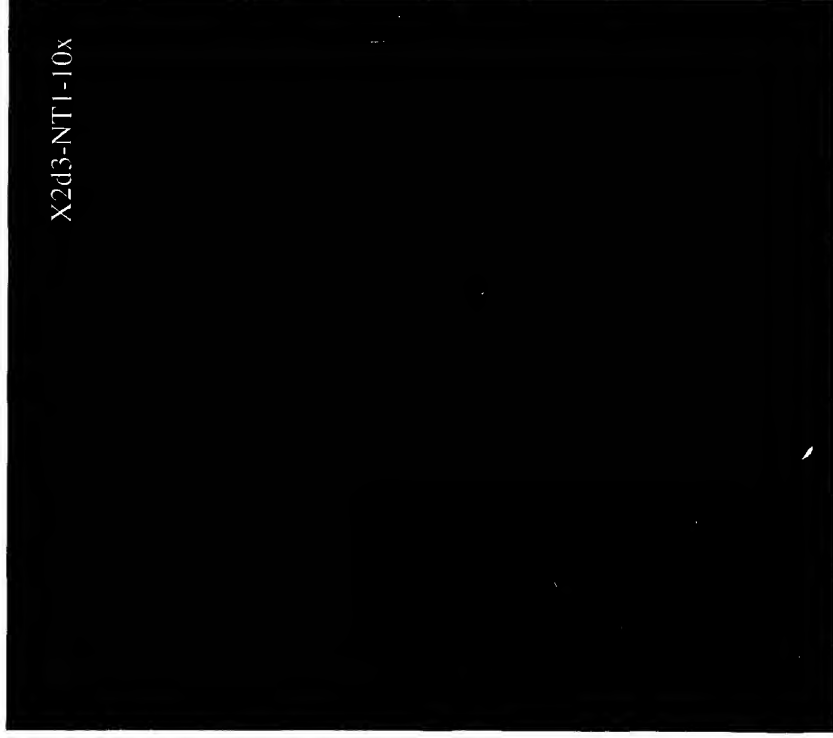


Fig.1b

Human MSCs in Mouse Muscle: Day-7

(H&E)



Dil labelled cells +
Sybr-green staining
of all nuclei

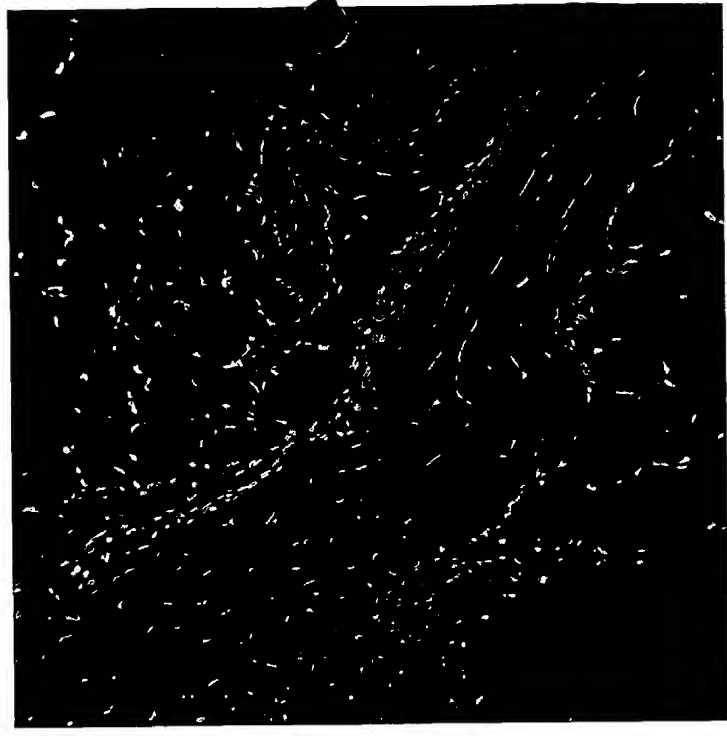


Fig.1c Human MSCs in Mouse Muscle: Day-7

(H&E, 20X)

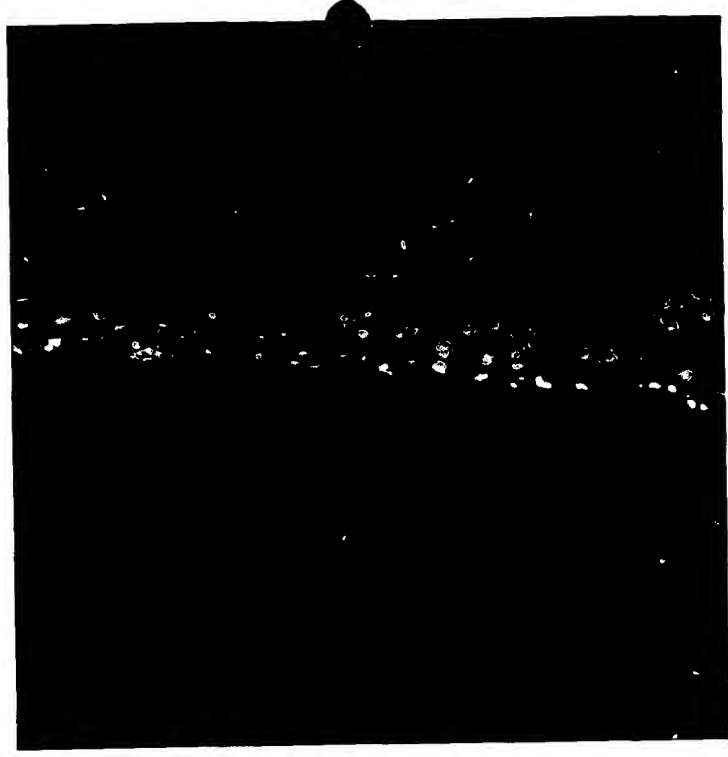
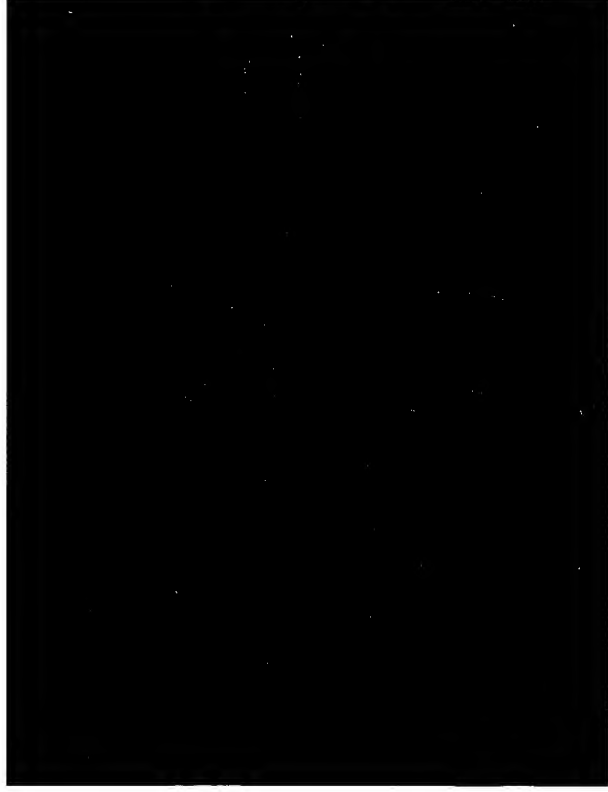
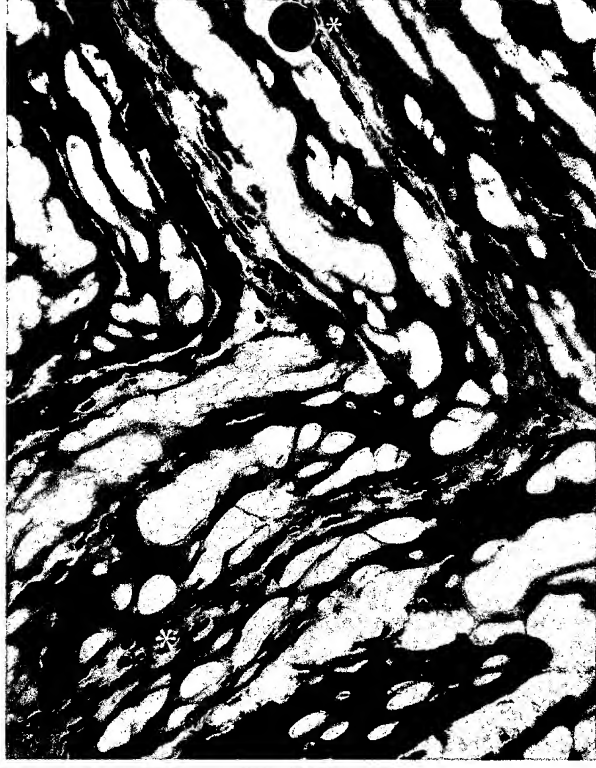


Fig.1d Human MSCs in Mouse Muscle: Day 14

Fluorescent Field, 10X



H&E 40X



**Fig. 1e DiI -Positive α -GAL Transduced hMSCs in
Immuno-competent mouse muscle**

d29



d43

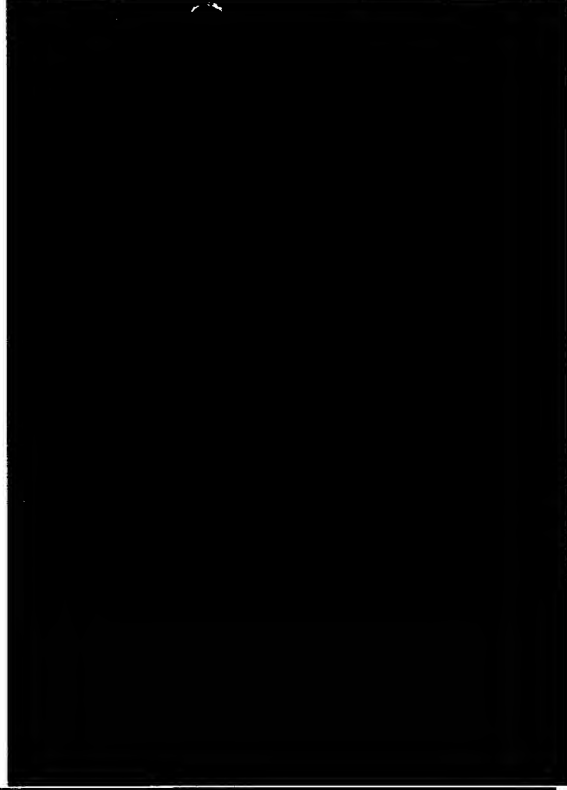
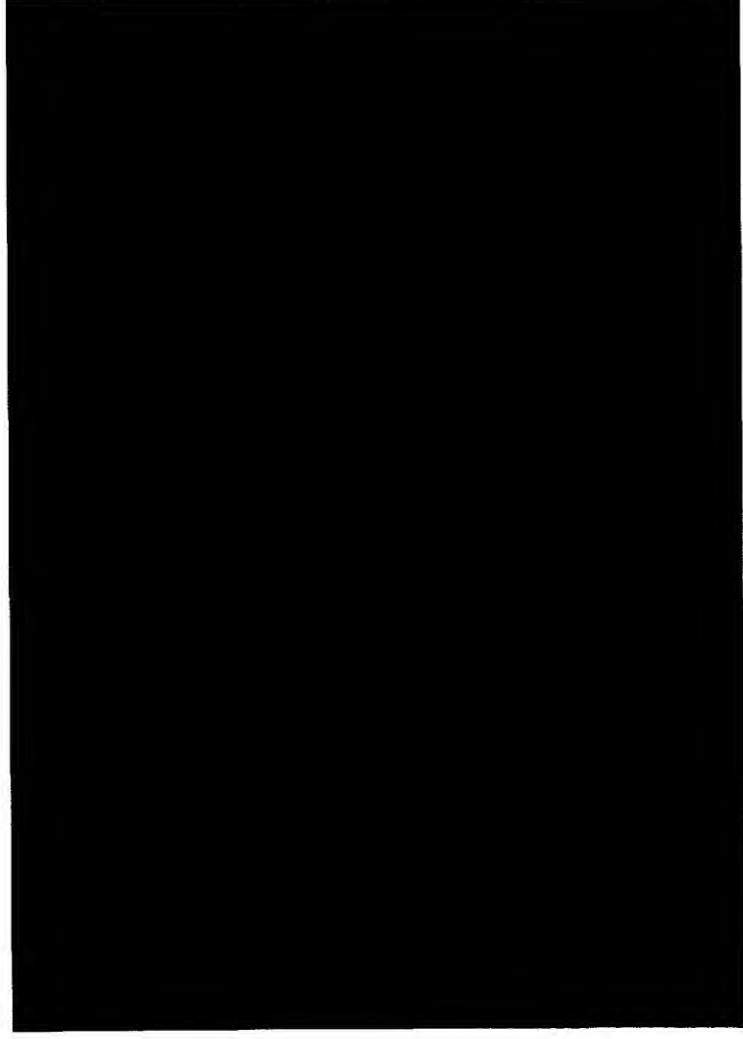


Fig.1f

Human α GalA-Transduced MSCs in Mouse Muscle: Day-57
10X



D3: IM injection of Dil-labeled PBMCs

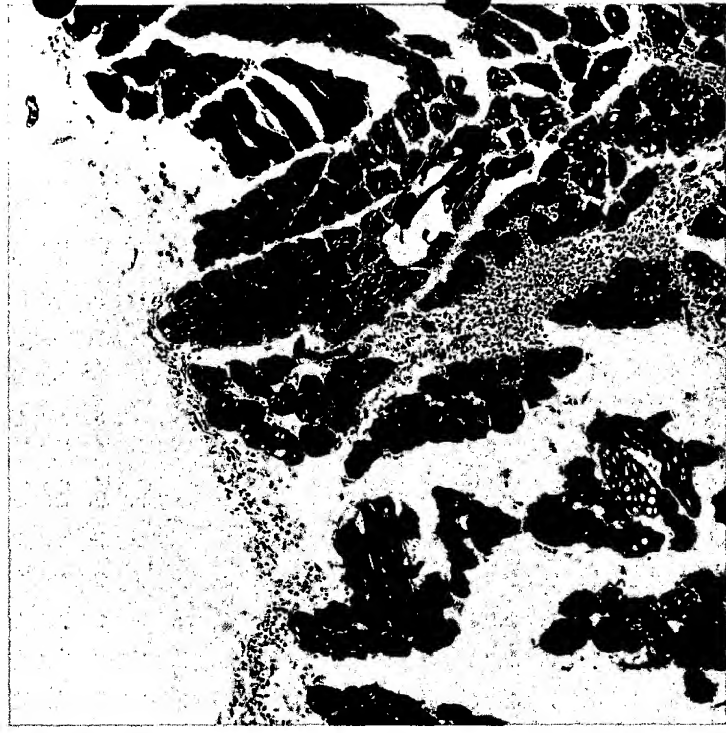
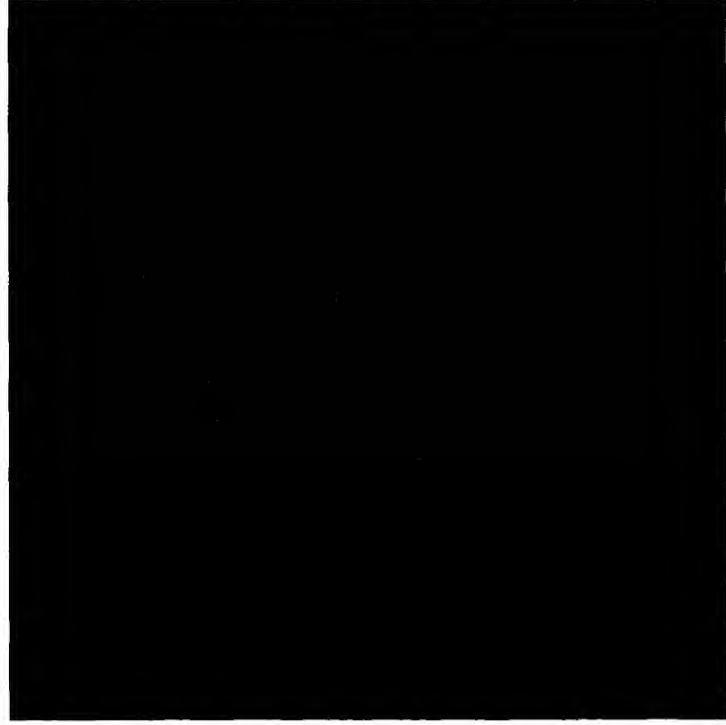
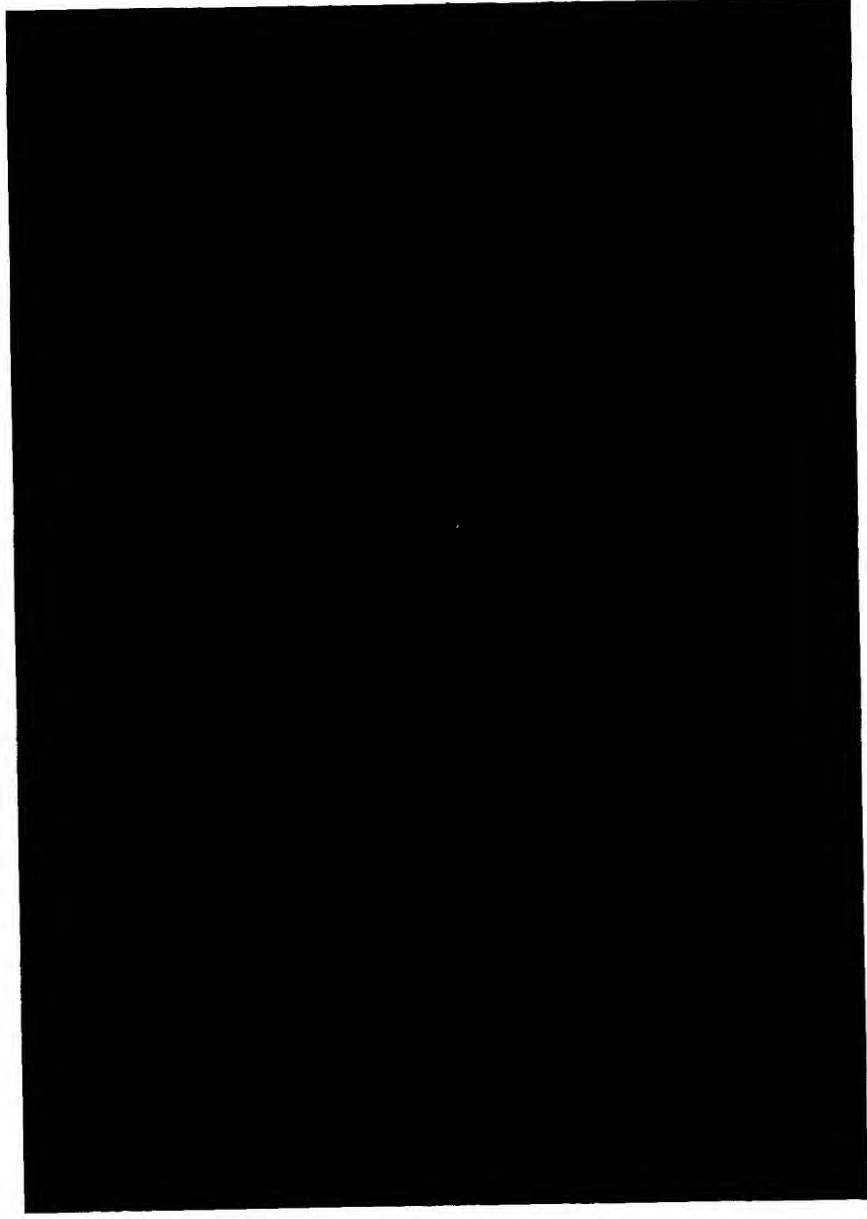


Figure-3

D3 IM injection of saline



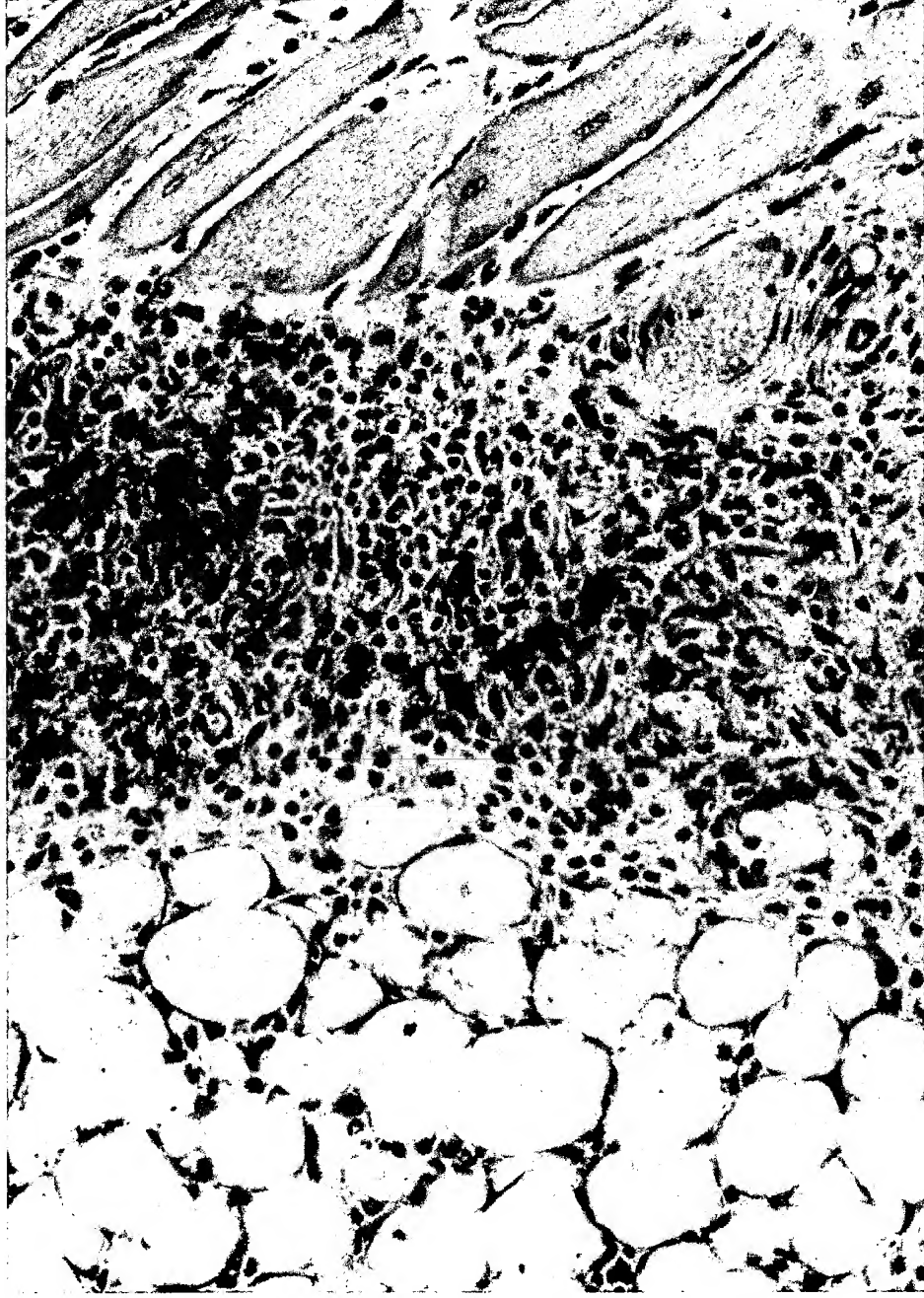
Fig. 4 6-week DAPI labeled hMSCs in Mouse Muscle



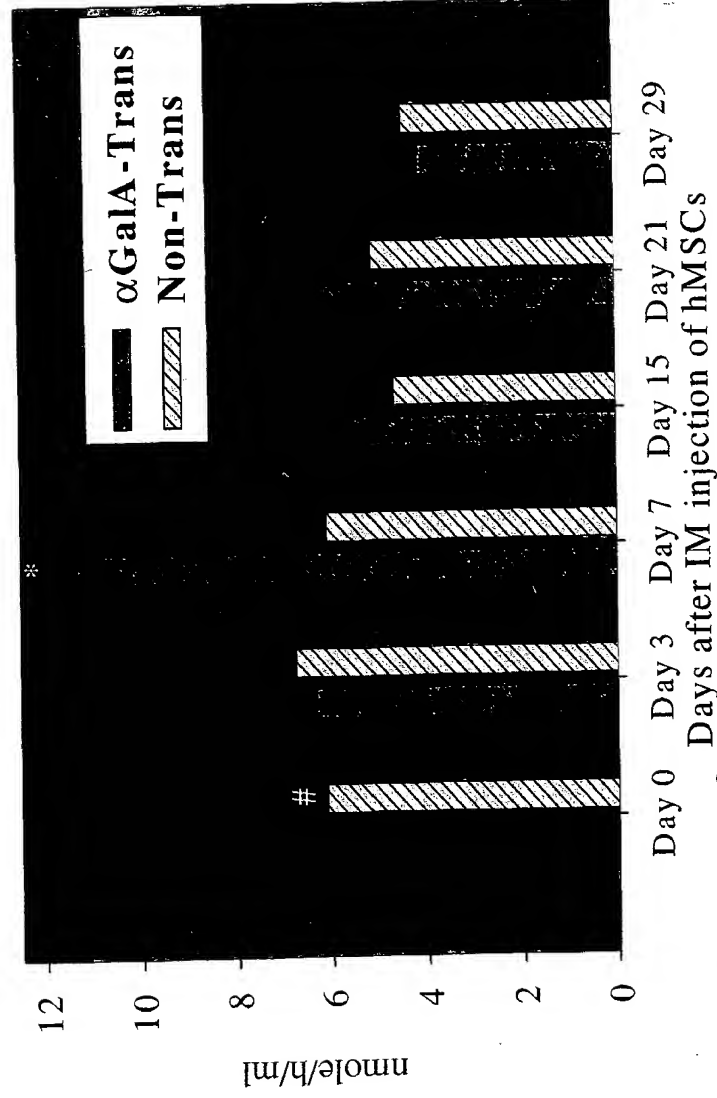
Red and Blue Fluorescence Channels merged

Fig.5

α GalA Immunostain of Muscle from Mouse
injected IM with α GalA-hMSCs: 14 days



α -Gala activity in Plasma of Mice IM-injected with α -Gala-Transduced hMSCs



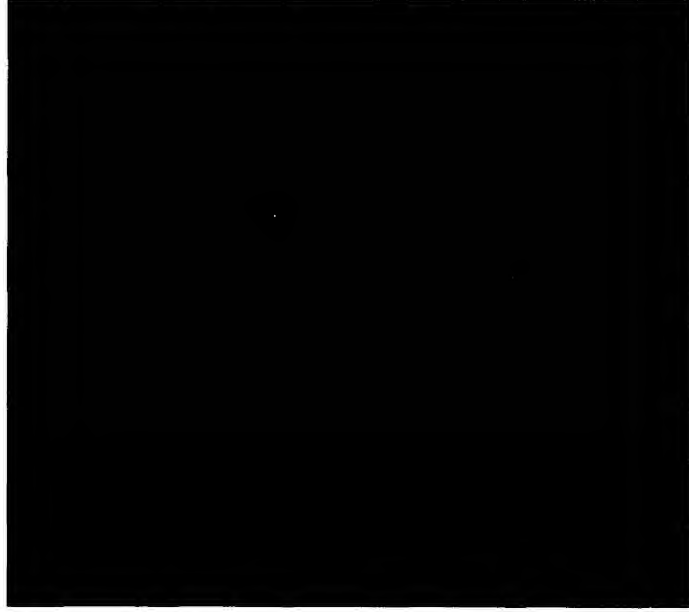
#: No MSCs, * n=2,

Fabry Mice plasma: 0.5 nmole/h/ml, PNAS 97:7515

Figure 7a

Human-specific Alu sequence staining of hMSCs injected into Immuno-competent mouse muscle

Dil-positive cells

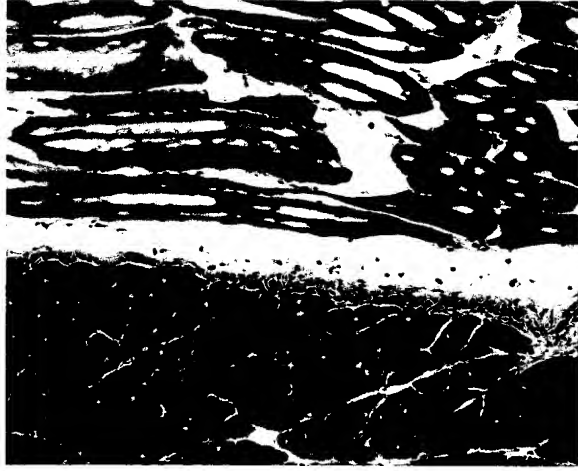


Alu-sequence positive cells

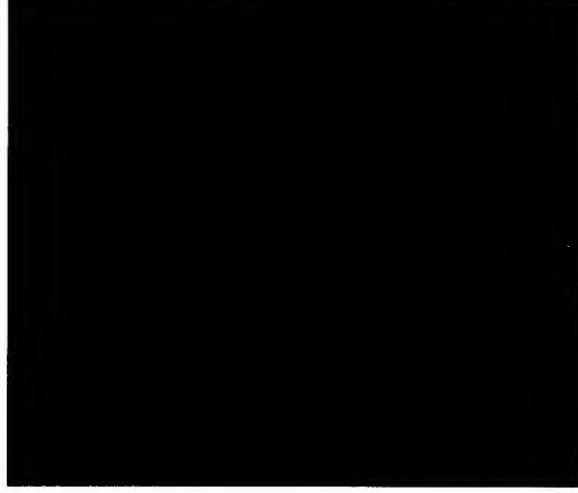


Human MSCs in Mouse Muscle: 43 days

H & E



Dil Positive Cells



Alu-Positive cells



Figure 7c

Day 57 DiI/Alu

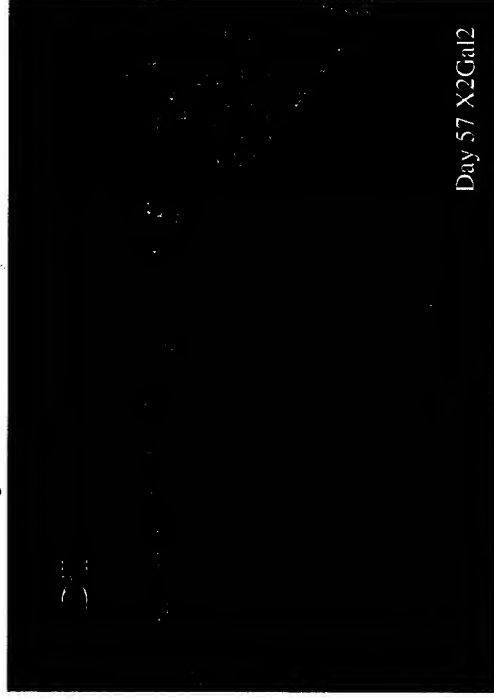
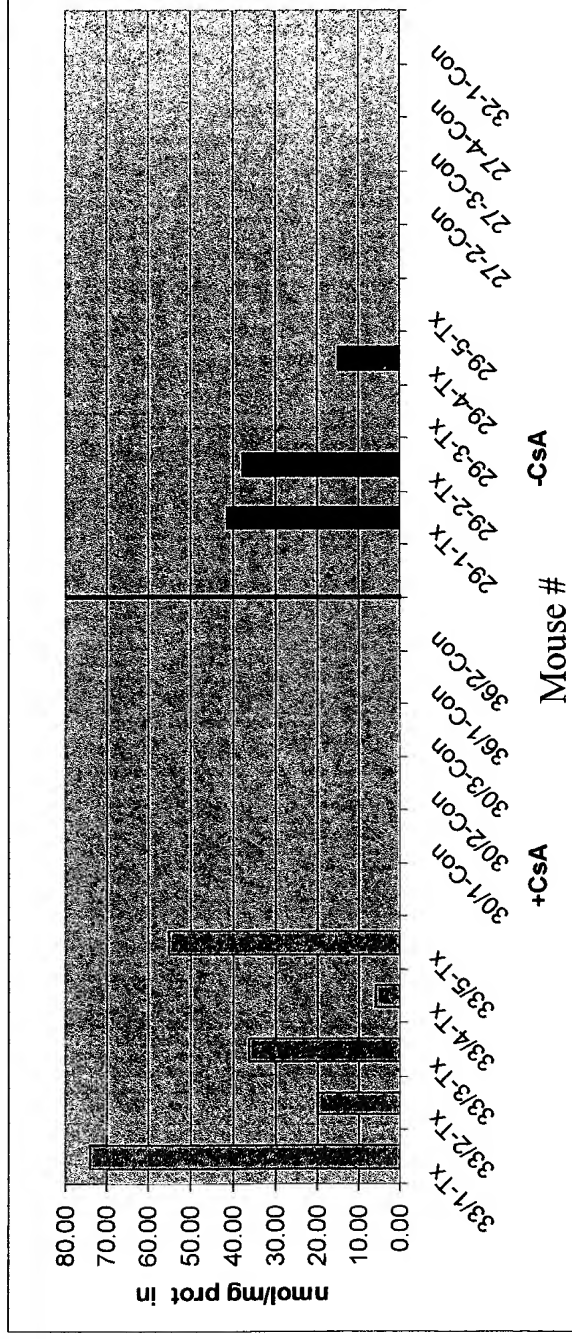


Figure-8a

α GalA Activity in Muscles of Fabry-KO mice:
14 days after IM injection of α GalA-Transduced hMSCs

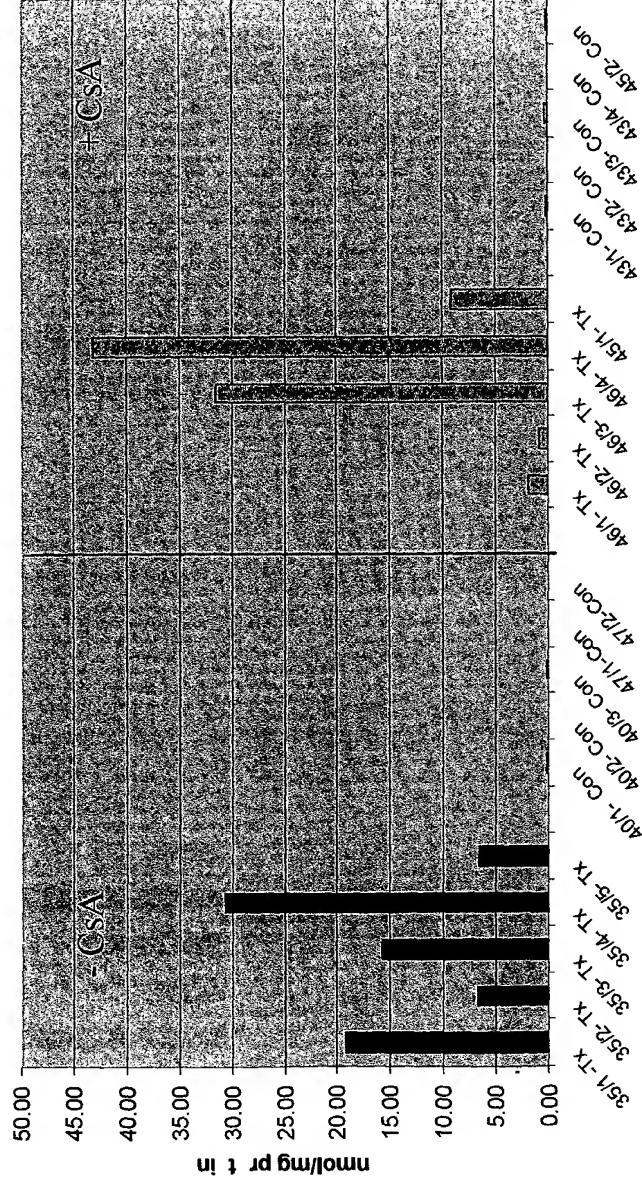


Tx = α GalA Transduced cells injected into muscle

Con = Vehicle alone injected into muscle

Figure 8b

**αGala Activity in Muscles of Fabry-KO mice:
28 days after IM injection of αGala-Transduced hMSCs**



Tx = αGala Transduced cells injected into muscle

Con = Vehicle alone injected into muscle

Figure-9a

FOXP2^{Cre} Liver from KO Mouse
 α GAL Immunostain of Liver from KO Mouse
injected IP with α GALA-hMSCs on Cultisphers :
14 days

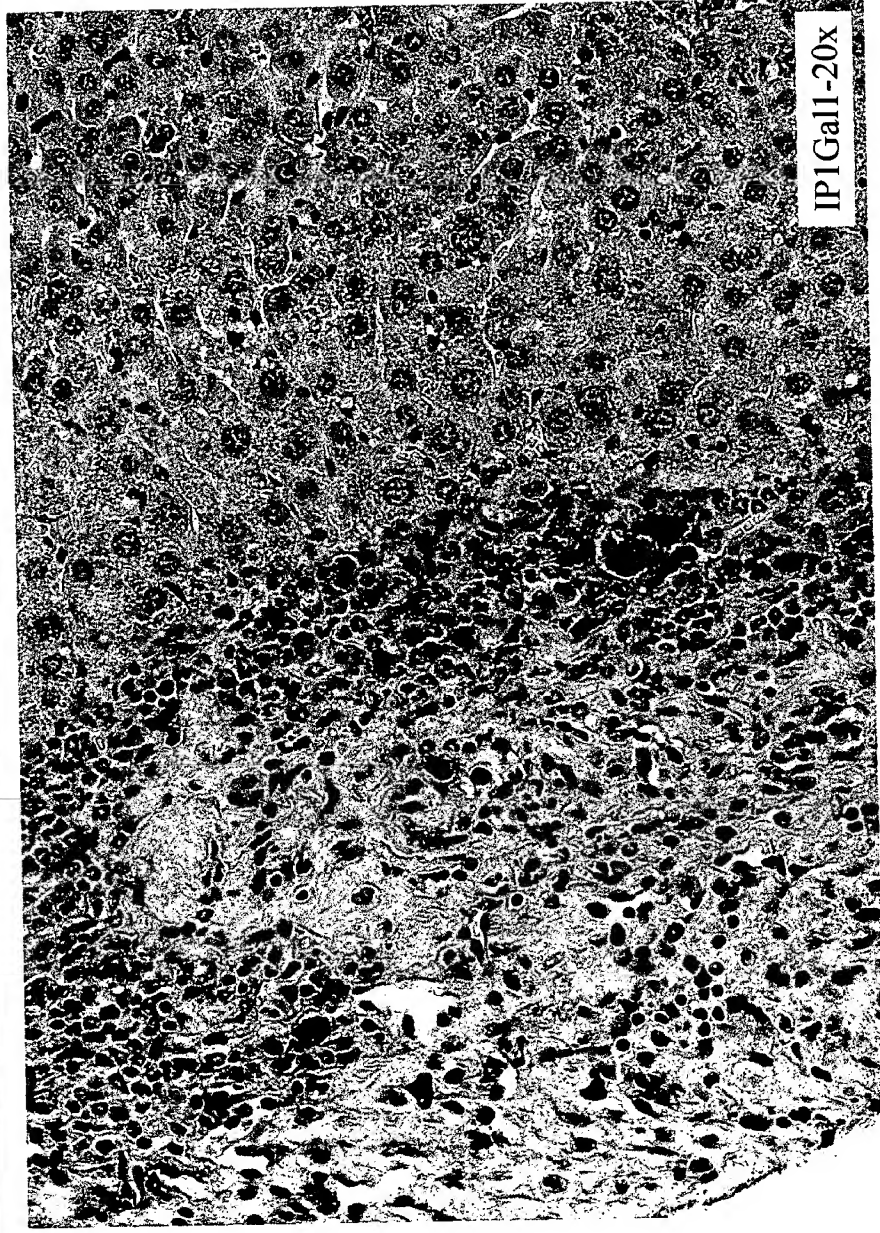


Figure 4b

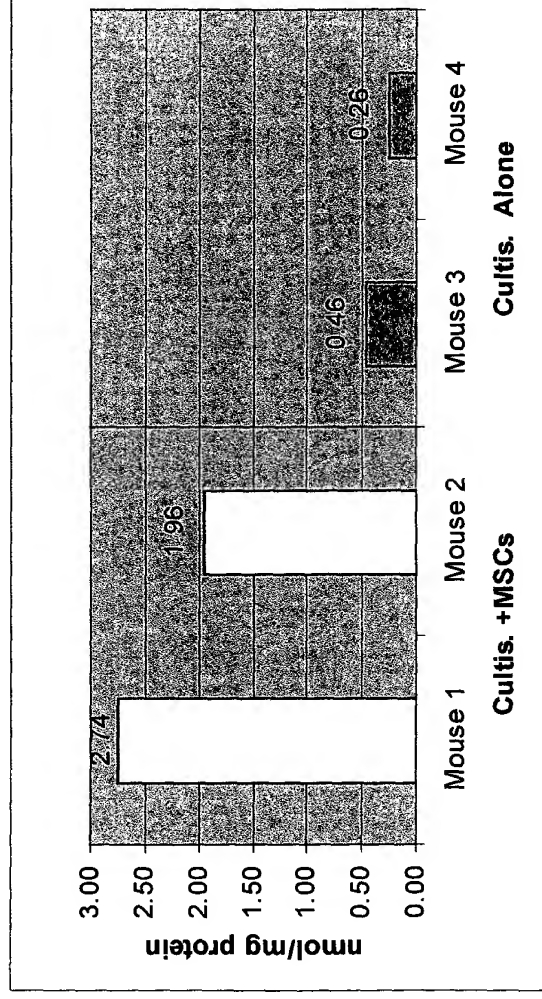
Pattern of Liver from KO Mouse
 α GalA Immunostain of Liver from KO Mouse
injected IP with α GalA-hMSCs on Cultispheres :
14 days



IP2Gal1 a-20x

Intra-Peritoneal Delivery of α GalA-Transduced hMSCs to Fabry Knock-Out Mice

α GalA Enzyme



Increase in α GalA is 5% of normal mouse liver activity (41 nmole/mg)